
RNA-directed therapy for Huntington's disease

Grant Award Details

RNA-directed therapy for Huntington's disease

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-13102

Investigator:

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| Name: | Eugene Yeo |
| Institution: | University of California, San Diego |
| Type: | PI |

Award Value: \$1,408,923

Status: Pre-Active

Grant Application Details

Application Title: RNA-directed therapy for Huntington's disease

Public Abstract: **Research Objective**

We develop a novel adeno-associated viral (AAV) vector-delivered RNA-targeting therapeutic for elimination of toxic RNA causative of Huntington's disease.

Impact

There are no disease-modifying therapies for Huntington's disease. Our therapeutic, if successful, will be a first-in-class treatment for this invariably fatal neurodegenerative disorder.

Major Proposed Activities

- In vitro studies of the RNA-targeting system in human Huntington's disease patient stem cell derived striatal organoids to assess the ability to eliminate toxic RNA foci
- AAV vector packaging of the CAG-targeting RNA-targeting system to obtain high-titer viral preparations, and in vivo (mouse) safety studies to assess immunogenicity, cytotoxicity and off-target effects
- In vivo efficacy studies of the RNA-targeting system in a mouse model of Huntington's disease to assess effects on disease-relevant molecular, cellular, behavioral and motor function deficits

Statement of Benefit to California: Currently, there is no cure for Huntington's disease, which currently affects thousands of Californians. The California population will equitably benefit from the development of a therapeutic for Huntington's disease, which affects the state's residents roughly equally across gender, race/ethnicity and socioeconomic status. Our therapeutic strategy is readily transferrable to a large set of other devastating diseases, multiplying the benefits of development of this new therapeutic modality.

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